Mass General Brigham
Health Plan

Galafold (migalastat)
Effective 12/1/2019

Plan	☐ MassHealth UPPL ☒Commercial/Exchange	**Program Type**	☒ Prior Authorization ☐ Quantity Limit ☐ Step Therapy
Benefit	☒ Pharmacy Benefit ☐ Medical Benefit		
Specialty Limitations	This medication has been designated specialty and must be filled at a contracted specialty pharmacy.		
Contact Information	**Medical and Specialty Medications**		
	All Plans	Phone: 877-519-1908	Fax: 855-540-3693
	Non-Specialty Medications		
	All Plans	Phone: 800-711-4555	Fax: 844-403-1029
Exceptions	N/A		

Overview

Migalastat is FDA indicated for Fabry disease. It is an oral pharmacological chaperone that stabilizes certain mutant variants of alpha-galactosidase to increase enzyme trafficking to lysosomes. Migalastat reversibly binds to the active site of the alpha-galactosidase A (alpha-Gal A) protein (encoded by the galactosidase alpha gene, GLA), which is deficient in Fabry disease. Binding to the active site stabilizes alpha-Gal A allowing trafficking from the endoplasmic reticulum into the site of action, the lysosome.

Coverage Guidelines

Authorizations will be granted for members who are currently receiving treatment with Galafold, excluding when the product is obtained as samples or via manufacturer's patient assistance programs

OR

Authorizations will be granted if the member meets all the following criteria and documentation has been submitted:

1. The member is at least 18 years of age
2. The member is diagnosed with Fabry disease
3. The prescriber is a clinical genetics specialist or nephrologist or consult with either specialist is provided
4. Results from enzyme assay test showing reduced or absent α-galactosidase A (α-GAL) enzyme activity in plasma, leukocytes, tears, or biopsied tissue are submitted
5. The member has GLA mutations which are amenable to treatment with Galafold

Limitations

1. Approvals will be granted for 12 months
2. Galafold will not be authorized in combination with enzyme replacement therapy (ERP)

References

1. Galafold (migalastat) [prescribing information]. Cranbury, NJ: Amicus Therapeutics US Inc; March 2020
2. Germain DP, Hughes DA, Nicholls K, et al. Treatment of Fabry's disease with the pharmacologic chaperone migalastat. *N Engl J Med*. 2016;375(6):545-555

3. Hopkin RJ, Jefferies JL, Laney DA, et al. The management and treatment of children with Fabry disease: A United States-based perspective. Mol Genet Metab 2016; 117:104
4. Terryn W, Cochat P, Froissart R, et al. Fabry nephropathy: indications for screening and guidance for diagnosis and treatment by the European Renal Best Practice. Nephrol Dial Transplant 2013; 28:505
5. McCafferty EH, Scott LJ. Migalastat: A Review in Fabry Disease. Drugs 2019; 79:543
6. Benjamin ER, Della Valle MC, Wu X, et al. The validation of pharmacogenetics for the identification of Fabry patients to be treated with migalastat. Genet Med 2017; 19:430

Review History

09/18/19 – Reviewed
09/16/20 – Reviewed at P&T.